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Father's Gift to Vite Lab is Personal

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FATHER'S GIFT TO VITE LAB IS PERSONAL

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SOON AFTER GALYA CHAN WAS BORN, HER LIVER BEGAN GROWING AT AN ALARMING RATE—SO QUICKLY THAT BY THE TIME SHE TURNED ONE, IT WAS THE SIZE OF A 13-YEAR-OLD'S. WITH DOCTORS UNABLE TO IDENTIFY THE CAUSE, GALYA SPENT THE BETTER PART OF HER FIRST YEAR IN AN INTENSIVE CARE UNIT.

After 12 frustrating and frightening months, the Chans finally received a diagnosis of Niemann-Pick type C disease (NPC), a rare, incurable childhood disease caused by the accumulation of excessive amounts of cholesterol and glycosphingolipids in the brain and other organs. Without FDA-approved treatments, and with only drugs to manage symptoms, young people with the degenerative condition often die before the age of 20.

"NPC is so rare that in all of Los Angeles, where we live, there are only four known cases," says Brian Chan, Galya's dad. "I've spent hundreds of hours researching it and talking to other families. As most NPC families do, I eventually ran into Dr. Vite at Penn Vet."

Dr. Charles H. Vite, Associate Professor of Veterinary Neurology, is part of a collaborative community of researchers around the world who are tackling NPC. Recently, he was the lead author of a study published in *Science Translational Medicine* that finds cats with NPC show vast improvement when treated with a compound called cyclodextrin. While NPC, which naturally occurs in cats and follows the same progression as it does in children, typically results in inexorable neurological decline, administering cyclodextrin into the fluid around the cats' brains largely halted the disease's progression. This study paved the way for the FDA to approve a Phase I clinical trial in children. A Phase 2/3 trial began this past fall.

For the Chans and other parents of children with NPC, breakthroughs like this offer hope. Determined to maintain forward momentum, they have become

active participants in NPC research, getting to know scientists and raising funds to keep studies moving.

Brian Chan is the founder of Liferay, Inc., an open source software company, and part of SOAR-NPC (Support of Accelerated Research for NPC), a scientist-clinician-patient advocate collaborative. After visiting Dr. Vite's lab with a group of parents, Chan asked what was needed to accelerate research. "A new microscope was high on the list," Chan says.

Without hesitation, he offered to support the purchase of a state-of-the-art microscope that will greatly enhance Penn Vet's ability to study NPC. Features like whole-slide scanning and 3-D reconstruction; digital archiving and sharing functions; high-resolution and live-cell, real-time imaging; and multiplexing capabilities will enable a better understanding of NPC's process. As a result, researchers can spend more time analyzing data and less time imaging it.

"We are grateful to Brian for this gift, and thankful for all of the determined, amazing families behind NPC research," says Vite. "They alone have helped fund 20 percent of our lab. We have essential NIH funding—we couldn't get by without it—but what really pushes things along is the involvement of the parents."

Regarding this unique partnership between families and researchers, Chan adds, "Doctors and scientists take an academic approach. For parents, it's personal. If you add a personal touch to scientific rigor, the results are better than if you have just academic data or parental passion. When you combine both… bam, things happen."

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—Dr. Charles H. Vite